HSCT for Multiple Sclerosis FAQ

All your questions about HSCT, AHSCT and how it relates to Multiple Sclerosis answered. Covering: what the HSCT treatment involves, the pros/cons and risks, the eligibility criteria for HSCT on the NHS, plus other non NHS options for HSCT and much more: –

1. What is HSCT for MS (Multiple Sclerosis)?

HSCT is a Haematopoietic Stem Cell Transplant; a procedure that can be used in
some Multiple Sclerosis sufferers.

2. How Does HSCT work?

HSCT involves aggressive chemotherapy and the use of a patient's own stem cells to reset the immune system.

The aim of the chemotherapy is to wipe out the harmful, faulty immune system cells that are attacking the brain and spinal cord. This is followed by re-infusion of the patient's previously collected haematopoietic stem cells to help regrow a new immune system for the patient.

The overall aim is to stop the immune system from ongoing attack of the spinal cord and brain, therefore preventing further neurological damage.

3. Can HSCT cure MS?

The purpose of HSCT for MS is not to cure all existing symptoms, but rather to stop the disease from progressing – studies so far show that it will do this for the overwhelming majority of patients.

4. What are the benefits of HSCT?

In HSCT clinical trials so far, the results suggest that HSCT may reduce relapses. It is also shown to stabilise, or in some cases reduce, the disability level for some relapsing remitting multiple sclerosis patients.

The biggest impact and the best response to the treatment was for patients with highly active relapsing-remitting MS. For the patients in this

group that the clinical trials have followed for four years, 80% had no more relapses and the disability showed no sign of getting worse for 87%.

In some cases the clinical trial results were more varied, for those with primary and secondary progressive MS the results are less effective. The results did not appear to be as effective for those with secondary progressive Multiple Sclerosis and those who had had relapsing remitting Multiple Sclerosis for more than ten years.

Source
<https://www.sth.nhs.uk/autologous-haematopoietic-stem-cell-transplantation-for-multiple-sclerosis/frequently-asked-questions.html#4>

5. How many times do I need to have HSCT?

At present, HSCT is a one off treatment. Studies are underway to monitor the results of patients who have undergone AHSCT. So far, the vast majority of patients show no further deterioration.

6. Is HSCT a good treatment for Multiple Sclerosis?

At present, HSCT is considered to be one of the best treatments for stopping further progression of MS. It is important to stress that every patient is different and each case has to be considered on an individual basis.

There are clinical trials being undertaken in the UK and around the world; these show very encouraging results, which you can read more about here. There are other treatments available and other research going on into Multiple Sclerosis at present.

7. What is the difference between AHSCT and HSCT?

When used in an MS context – nothing. The 'A' here stands for 'Autologous', which means that the stem cells come directly from the patient themselves. Stem cell treatments for other conditions may be 'Allogeneic', which means that they come from a donor.

8. What is the Difference between Myeloablative and Non-Myeloablative HSCT?

The goal of all HSCT therapy protocols for autoimmune conditions is to eliminate the faulty T and B cells which are responsible for the disease.

Myeloablative HSCT typically ablates a greater percentage of these cells than non-myeloablative HSCT, but non-myeloablative HSCT is still sufficient to bring the underlying activity to below the activation threshold.

Both approaches work for the overwhelming majority of recipients, but the long term results for either approach have yet to be formally compared. Non-myeloablative recipients typically have a more prompt

recovery profile, on average. UK, Mexico and Russia all have a non-myelo protocol, but they vary in intensity.

9. Can I get HSCT on the NHS?

You can, if you meet their criteria. You will require a referral from your Neurologist or your GP (in Scotland the referral must come from your Neurologist as Scotland is governed by different funding rules).

10. What are the criteria for HSCT on the NHS?

There are strict criteria for HSCT treatment for Multiple Sclerosis on the NHS, covering both the referral process and the inclusion criteria. There are also exclusion criteria covered in the next question.

NHS referral criteria:

- Diagnosis of MS made by a neurologist
- Able to walk, needing at most bilateral assistance to walk 20m without resting
- In relapsing MS (RMS), failed one licensed disease modifying drug of high efficacy (currently including alemtuzumab and natalizumab) because of demonstrated lack of efficacy
- New MRI activity within last 12 months

Inclusion criteria:

- Age 18 to 65 years
- Disease duration ≤15 years from diagnosis of MS
- Diagnosis of MS according to McDonald's criteria
- For PPMS, CSF OCB+
- For RMS, failed at least one licensed disease modifying drug of high efficacy ('Category 2' as defined by Scolding N, Barnes D, Cader S, et al. Pract Neurol 2015;15:273–279; currently including alemtuzumab and natalizumab) because of demonstrated lack of efficacy (as evident from relapse, MRI activity as defined below at

Point 7, or EDSS increase) after being on DMT for at least 6 months
- EDSS score 0-6.5
- Inflammatory active MS as defined by 1 Gd+ (>3mm) lesion (off steroids for one month) or 2 new T2 lesions in MRI within last 12 months
- Approved by the MDT (The HSCT Multi-Disciplinary Team)

Source - London MS-AHSCT Collaborative Group – Patient Eligibility Criteria Final V.3. – 8/12/2015

11. Are there any reasons I wouldn't get HSCT on the NHS?

The reasons why you wouldn't be able to take part in treatment on the NHS are covered below.

Exclusion criteria:

- Eligible for an ethically approved clinical trial where AHSCT is offered as one of the treatment arms

- Unable to adequately understand risk and benefits of AHSCT and give written informed consent
- Prior treatment with total lymphoid irradiation and autologous or allogeneic hematopoietic stem cell transplantation
- Contraindication to MRI including but not limited to metal implants or fragments, history of claustrophobia or the inability of the subject to lie still on their back
- Poorly controlled depression or recent suicidal attempt
- Presence of any active or chronic infection
- Unable to walk 20mt with or without support, or wheelchair dependent
- Any significant organ dysfunction or co-morbidity that the Investigators consider would put the subject at unacceptable risk

Source - London MS-AHSCT Collaborative Group – Patient Eligibility Criteria Final V.3. – 8/12/2015

12. What is the difference between an Active Lesion and a T2 Lesion?

An active lesion can be compared to a fresh wound or a scab. A T2 lesion is more like a scar.

An MRI can only pick up what it sees at the time of the examination, and as active lesions have a life of 30-40 days, it is therefore quite possible that new activity may be missed on an MRI – this is why the MDT also accept new T2 lesions (from MRI within 12 months of each other), as they show that there has been recent activity.

13. What does EDSS mean?

Expanded Disability Status Scale (EDSS) is a method of quantifying disability in MS: -

0.0: Normal Neurological Exam
1.0: No disability, minimal signs in 1 FS
1.5: No disability, minimal signs in more than 1 FS
2.0: Minimal disability in 1 FS
2.5: Mild disability in 1 or Minimal disability in 2 FS
3.0: Moderate disability in 1 FS or mild disability in 3 - 4 FS, though fully ambulatory
3.5: Fully ambulatory but with moderate disability in 1 FS and mild disability in 1 or 2 FS; or moderate disability in 2 FS; or mild disability in 5 FS
4.0: Fully ambulatory without aid, up and about 12hrs a day despite relatively severe disability. Able to walk without aid 500 meters
4.5: Fully ambulatory without aid, up and about much of day, able to work a full day, may otherwise have some limitations of full activity or require minimal assistance. Relatively severe disability. Able to walk without aid 300 meters
5.0: Ambulatory without aid for about 200 meters. Disability impairs full daily activities
5.5: Ambulatory for 100 meters, disability precludes full daily activities

6.0: Intermittent or unilateral constant assistance (cane, crutch or brace) required to walk 100 meters with or without resting
6.5: Constant bilateral support (cane, crutch or braces) required to walk 20 meters without resting
7.0: Unable to walk beyond 5 meters even with aid, essentially restricted to wheelchair, wheels self, transfers alone; active in wheelchair about 12 hours a day
7.5: Unable to take more than a few steps, restricted to wheelchair, may need aid to transfer; wheels self, but may require motorized chair for full day's activities
8.0: Essentially restricted to bed, chair, or wheelchair, but may be out of bed much of day; retains self care functions, generally effective use of arms
8.5: Essentially restricted to bed much of day, some effective use of arms, retains some self care functions
9.0: Helpless bed patient, can communicate and eat
9.5: Unable to communicate effectively or eat/swallow
10.0: Death due to MS

14. Where else can I get HSCT?

There are a number of approved HSCT centres across the world however, this charity concentrates specifically on providing support and advice for patients having HSCT in the UK, Mexico (Clinica Ruiz) and Russia (Maximov Hospital).

15. How much does HSCT cost privately / overseas?

Treatment ranges from £30,000 to just under £110,000 (exchange rate correct on 20/07/18). Some facilities include outpatient treatment and accommodation for a patient and carer, with other centres following an inpatient protocol. Travel expenses are not included.

16. But I've seen a Stem Cell Therapy being offered for a LOT less than that!

It is important that prospective patients are diligent in their research and check any centre thoroughly.

The centre should be on the Joint Accreditation Committee-ISCT & EBMT (JACIE) accredited or equivalent.

Typically, there are facilities across the world offering 'Stem Cell Therapy' or 'Stem Cell Treatment' for MS – more often than not, this is not HSCT. We caution: no chemo; no cure. There is no current scientific research to support the halting of the progression of MS with stem cells alone. AIMS currently only endorses the two following international hospitals, which are highly experienced with treating international MS / Autoimmune patients with HSCT: Clinica Ruiz, Mexico and AA Maximov Hospital, Moscow.

17. My Neurologist says that HSCT is very dangerous, with a high mortality rate – is this true?

At the International HSCT Symposium held in the UK on Friday October 14th 2016, a leading HSCT Specialist (Dr Richard K Burt, who ran the third phase international HSCT trials based in Chicago) stated that the risk of HSCT for MS is below 0.5% in early relapsing MS (RMS).

In actual fact, more people are killed in hippo attacks every day than have ever died from HSCT. Approved HSCT facilities take great care to

ensure that patients are fit for treatment. HSCT is still a relatively unfamiliar concept to a lot of neurologists, as it is a haematologist's specialism, and they are not always in full possession of the facts.

18. My Neurologist says HSCT is only for people with RRMS, but I have PPMS / SPMS / RPMS - does this mean I'm not suitable?

Patients with all forms of MS are treated on the NHS, providing they meet the additional criteria.

While RRMS patients stand the best chance of seeing their symptoms reversed (given the fact that the disease may not have had as much opportunity to cause as much permanent damage), all patients stand a very good chance of having their progression halted, with many progressive patients also reporting symptomatic improvements. If you do not meet the NHS criteria, overseas centres may still be suitable for you.

19. I'm in a wheelchair – will HSCT allow me to walk again?

In some cases, the damage done by MS may be irreversible, and there is no guarantee of any symptomatic improvement. However, there have been cases of both RRMS and progressive MS patients regaining some mobility. It should be stressed that advanced disability is unlikely to respond and may increase the risk of the HSCT procedure.

20. I've had MS for over 20 years – is it too late for me to have HSCT?

In the UK, yes, but a diagnosis over 15 years will not preclude you from receiving HSCT at other approved centres overseas. Similarly, some international centres will accept patients with an EDSS higher than 6.5 (UK maximum).

21. Will HSCT help my other MS Symptoms?

It may do, but the aim is to stop progression, and this is likely in the overwhelming majority of patients, irrespective of disease form, duration or EDSS.

22. Will I lose my fertility / risk secondary cancers from HSCT?

The risk of post-non-myeloablative HSCT secondary cancer is so low that it cannot be expressed as a meaningful non-zero number.

For females that don't regain hormone function post-HSCT, this is treatable with daily medication. The risk of experiencing infertility in non-myelo HSCT is age dependent with females below the age of 30 unlikely to become permanently infertile, but this risk increases with age over 30.

Anyone wishing to preserve their fertility should plan ahead in case a later IVF procedure is required. HSCT will not prevent a woman from having a healthy pregnancy or from bringing a baby to a healthy full term delivery.